

(19) World Intellectual Property
Organization
International Bureau



543 033

(43) International Publication Date
5 August 2004 (05.08.2004)

PCT

(10) International Publication Number
WO 2004/065561 A2

- (51) International Patent Classification⁷: C12N (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (21) International Application Number: PCT/US2004/001643
- (22) International Filing Date: 21 January 2004 (21.01.2004)
- (25) Filing Language: English
- (26) Publication Language: English
- (30) Priority Data: 60/441,637 21 January 2003 (21.01.2003) US
- (71) Applicant (for all designated States except US): PTC THERAPEUTICS, INC. [US/US]; 100 Corporate Court, South Plainfield, NJ 07080 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): CAO, Liangxian [CN/US]; 6 Mioduski Court, Parlin, NJ 08859 (US). TRIFILLIS, Panayiota [US/US]; 309 Rivercrest Drive, Piscataway, NJ 08854 (US).
- (74) Agents: CORUZZI, Laura, A. et al.; Jones Day, 222 East 41st Street, New York, NY 10017-6702 (US).
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

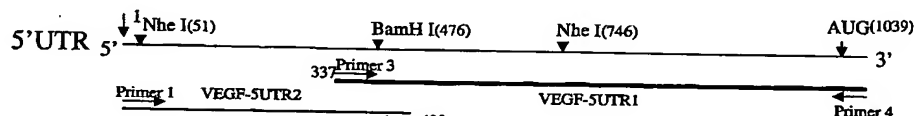
Published:

— without international search report and to be republished upon receipt of that report

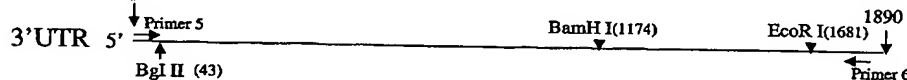
[Continued on next page]

(54) Title: METHODS FOR IDENTIFYING COMPOUNDS THAT MODULATE UNTRANSLATED REGION-DEPENDENT GENE EXPRESSION AND METHODS OF USING SAME

A.



B.



C.

Primer 1: 5'-AAAGTCGACGTAATCGCGGAGGCTTGGGCAGCCGG-3'

Primer 2: 5'-TTTGCGACTGGTCAGCTGCGGGATCCCAAG-3'

Primer 3: 5'-AAGTCGACGTAAGAGCTCCAGAGAGAAGTCGAG-3'

Primer 4: 5'-AAACCCGGGCAGCAAGGCAAGGCTCCAATGCAC-3'

Primer 5: 5'-GCCGGGCAGGAGGAAGGAGCCTCCCTCAGGGTTTGGGGA-3'

Primer 6: 5'-CTGCACTAGAGACAAAGACGTGATGTTAAT-3'

(57) Abstract: The present invention relates to methods for identifying compounds that modulate untranslated region-dependent expression of a target gene. The invention particularly relates to using untranslated regions of a target gene or fragments thereof linked to a reporter gene to identify compounds that modulate untranslated region-dependent expression of a target gene. The methods of the present invention provide a simple, sensitive assay for high-throughput screening of libraries of compounds to identify pharmaceutical leads.



For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.